

Investor Presentation

November 2021



Forward-Looking Statements



This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. These statements may be identified by the words “may,” “will,” “could,” “would,” “should,” “expect,” “plan,” “anticipate,” “intend,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “target,” or other similar terms or expressions that concern X4’s expectations, strategy, plans, or intentions. Forward-looking statements include, without limitation, statements regarding the clinical development and therapeutic potential of mavorixafor for the treatment of WHIM syndrome, Waldenström’s macroglobulinemia, congenital neutropenia and other neutropenias and other primary immunodeficiencies, and of X4’s other product candidates; X4’s possible exploration of additional opportunities for mavorixafor; the expected duration of patent protection; the expected availability, content and timing of clinical data from X4’s ongoing clinical trials of mavorixafor; anticipated regulatory filings; clinical trial design; and X4’s cash runway and ability to satisfy covenants in agreements with third parties.

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X4: Building On Success With Near-Term Catalysts; Delivering High Growth



Advancing innovative treatments for rare diseases caused by dysregulation of the immune system

Clinically proven Phase 3 candidate: Mavorixafor - a once-daily oral CXCR4 antagonist

Advancing Mavorixafor to potentially treat >30,000 patients (US and EU Markets) in multiple indications

Building a sustainable Rare Disease Company with a growing pipeline

Key Value Drivers

- ✓ Strong Phase 2 PoC; Breakthrough Therapy Designation in Lead Indication (WHIM syndrome)
- ✓ Fully Enrolled Global Phase 3 trial in WHIM
- ✓ Positive Phase 1b Biomarker Data in Waldenstrom's macroglobulinemia (lymphoma)
- ✓ Ongoing Phase 1b trial in Chronic Neutropenia
- ✓ Pipeline of multiple pre-clinical compounds

Expected Upcoming Milestones

4Q21: ASH and Investor Day

- Response Rates In Waldenstrom's Phase 1b
- Initial Data in Chronic Neutropenia
- Long-term Outcomes in WHIM Phase 2
- WHIM prevalence/patient ID update

2H22: Data & regulatory updates on CN/WM trials

2H22: New molecule entering the clinic

2H22: WHIM prevalence/patient ID updates

4Q22: Top-Line Phase 3 Data in WHIM

Broad Market Potential through CXCR4 Antagonism

Addressing the Needs of >30,000¹ patients in US and EU Markets in initial three indications

Candidate	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Expected Milestones	Target Patient Populations
Mavorixafor	WHIM (Warts, Hypogammaglobulinemia, Infections and Myelokathexis) syndrome ²	Phase 3				Top-line data 4Q 2022 NDA 1Q 2023	1,000-3,700 U.S. ³
	Waldenström's Macroglobulinemia (WM)	Phase 1b				Additional data in 4Q 2021	2,000-3,000 U.S. ⁴
	Chronic Neutropenia (CN) (Severe and Moderate)	Phase 1b				Initial data in 4Q 2021	5,000-10,000 U.S. ⁵
X4P-002	Oncology indications	IND-enabling				IND in 2H 2022	Other leukemias and lymphomas >25,000 ⁴
X4P-003	Primary immuno-deficiencies (PID)						Undisclosed

1. EU Estimates: using U.S. prevalence of each disease and applying it to EU population. 2. Phase 2 open label extension (OLE) trial for WHIM ongoing 3. Company market research. Qessential market research, 2019 and IPM.ai artificial intelligence study, 2020. 4. *WM Epidemiology Analysis Nemetz Group*. Data on file. 5. Estimate using Andersen et al. *J Intern Med*. 2016 Jun;279(6):566-75.

Seasoned Executive Leadership Team



PAULA RAGAN, Ph.D.
President & CEO

genzyme

SANOFI



DIEGO CADAVID, M.D.
Chief Medical Officer



Fulcrum
Therapeutics



Biogen



ADAM MOSTAFA
Chief Financial Officer

abpro

CANTOR
Pittsburgh



ART TAVERAS, Ph.D.
Chief Scientific Officer



Schering-Plough



Biogen



MARY DIBIASE, Ph.D.
Chief Operating Officer



Biogen



EPIRUS
BIOPHARMACEUTICALS



DEREK MEISNER, J.D.
Chief Legal Officer



genocea

RACapital

Dysregulation of white blood cells (leukocytes) contributes to a broad range of serious diseases with significant unmet needs



Primary Immunodeficiencies

Life-threatening infections & deaths

- >\$3.3 billion WW annual Rx sales¹
- Few options (G-CSF & IVIG)
- Injectable or infusion only



Lymphomas

Cancer progression and deaths

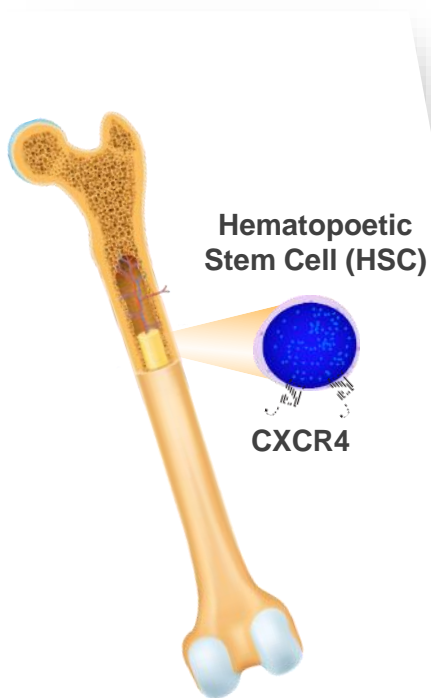
- >\$7 billion WW annual Rx sales²
- Multiple therapies and multiple lines of treatment
- Few cures

1. Allied Market Research 2019

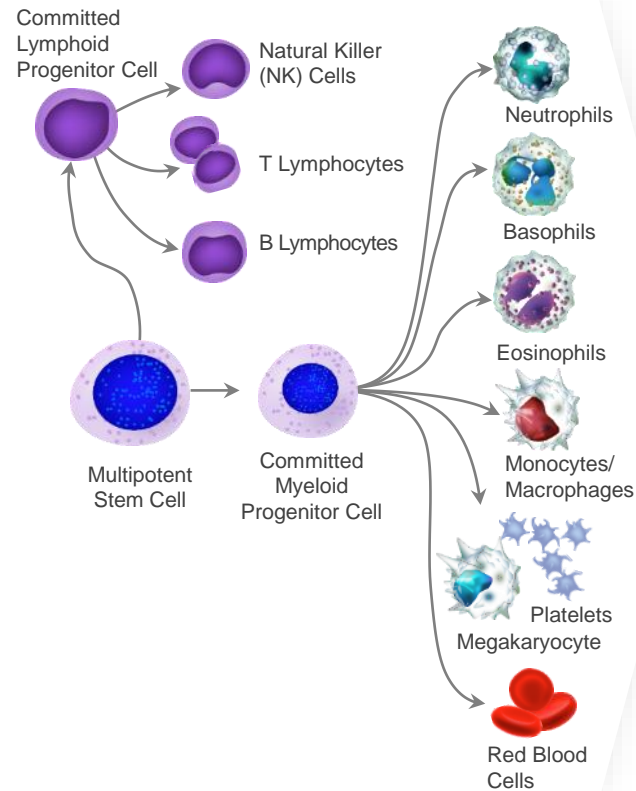
2. Business Insight, Market Research Report, 2018

CXCR4/CXCL12 Pathway: Master Regulator of Healthy Immune System Function

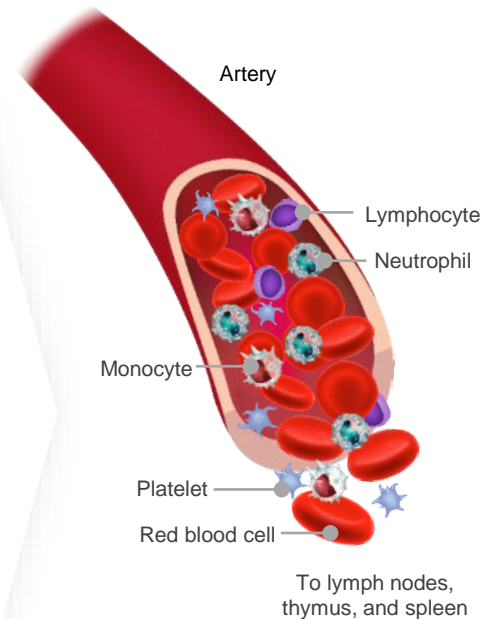
EXPANSION



MATURATION



TRAFFICKING



CXCR4 Antagonism Clinically Proven to:

- ✓ Increase maturation and mobilization of WBCs in all those dosed
- ✓ Increase circulating neutrophils, lymphocytes, and monocytes in patients with immunodeficiencies and certain cancers
- ✓ Reduce bacterial and viral infections
- ✓ Reduce lymphoma burden in combination with oncology Rx

CXCR4 Plays Key Role in Regulating Expansion, Maturation, and Trafficking of Leukocytes

Mavorixafor: Realizing the Potential of CXCR4 Antagonism in an Oral Capsule



The only oral CXCR4 antagonist in development

- Small molecule with high potency and selectivity
- Durable half-life supporting once daily dosing
 - 2 or 4 capsules, once per day (WHIM)

Antagonizes Both Wild-Type and Mutant CXCR4

- Broad applicability to various disease states with high unmet needs

Safety Profile Supports Chronic Use

- >200 patients/subjects treated to date
- Some patients on Rx for several years

Favorable Regulatory Designations (WHIM)

- Breakthrough Therapy Designation (U.S.)
- Fast Track Designation (U.S.)
- Rare Pediatric Disease Designation (PRV eligible)
- Orphan Drug Status in U.S. and Europe

Patent Protection Expected Through 2038 and Beyond



The Dysregulated Immune System: Immunodeficiencies

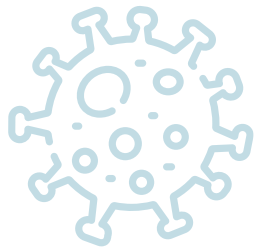
Immunodeficiencies (US Prevalence)



Combined Variable Immune Deficiency (CVID)¹
>10,000



Chronic Neutropenias²
5,000 - 10,000
(severe and moderate)



WHIM Syndrome³
~1,000 to 3,700

Chronic Immunosuppression

- Life-long severe and/or life-threatening infections
- Reduced/no response to vaccines
- Increased cancer risk
- High morbidity (bronchiectasis, hearing loss) and life-threatening sepsis

Caused By

- Low WBC counts (cytopenias) and/or
- Dysregulated or dysfunctional immune cells

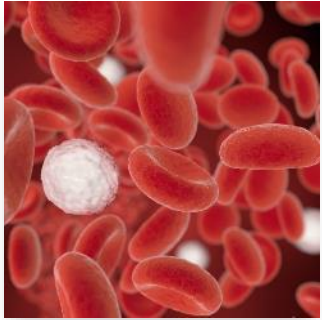
Treated With Injectables

- Antibiotics for acute infections
- G-CSF for those with severe neutropenia
- IVIG for those with hypogammaglobulinemia (prevention)
 - Stem cell transplant in rare/severe cases

Unmet need: Oral treatment that corrects and regulates a range of immune system deficiencies

Introduction to WHIM Syndrome

An Immunodeficiency Affecting Children and Adults



Pancytopenia: All White Blood Cells Affected & Reduced



Severe bacterial infections

In multiple organ systems: bronchiectasis (lung), hearing loss (ear), cellulitis (skin)



Viral infections & cancer risk

Disfiguring recalcitrant warts; EBV and HPV-associated cancers

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Approved targeted therapies; symptomatic treatment with G-CSF and IVIG

www.whimsyndrome.com

Leanne's Story - *Living with WHIM Syndrome*



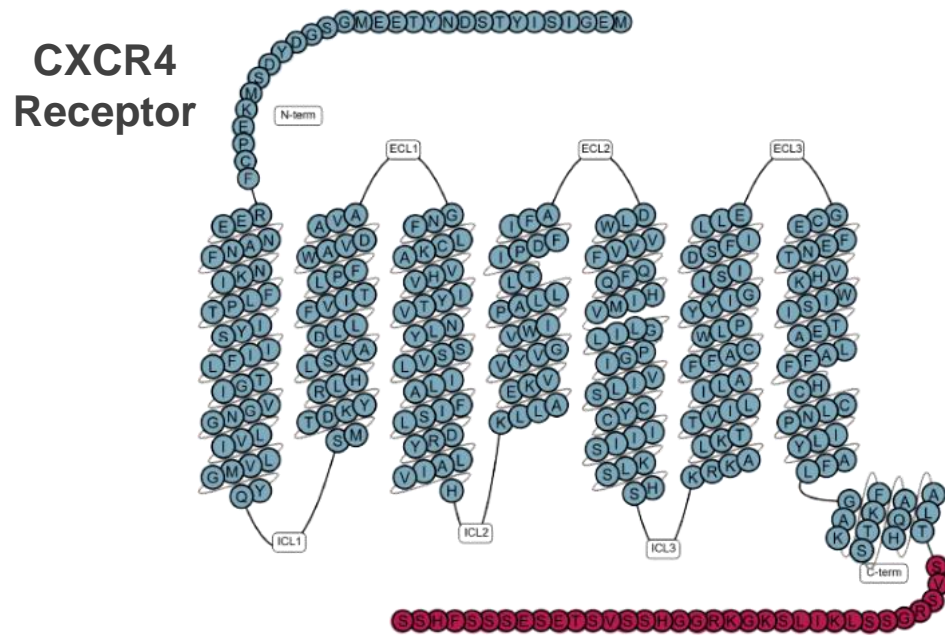
WHIM Patient
New South Wales, Australia

[In early marriage], I had 8 episodes of pneumonia in 8 months, more likely one continual episode of pneumonia. I had a picc line, I was having intravenous antibiotics.

Every time I was better, they'd stop the antibiotics and almost immediately I'd become unwell again.

Root Cause of WHIM Syndrome: Over-Signaling of CXCR4 due to Genetic Mutations

**Mutations
cause over-signaling**



**Impacting immune cell expansion,
maturation, and trafficking**

- **WHIM Syndrome:** spectrum of clinical presentations mostly with (and sometimes without) CXCR4 mutations
 - **W:** warts
 - **H:** hypogammaglobulinemia
 - **I:** infections
 - **M:** myelokathexis (hyper-cellular bone marrow)
- In most cases, **autosomal dominant disease** driven by pathogenic CXCR4 mutations; mostly located in "tail" (c-terminus) of receptor
 - ~16 identified to date; # increasing with research
- Results in **over-signaling of CXCR4 impacting all white blood cells**

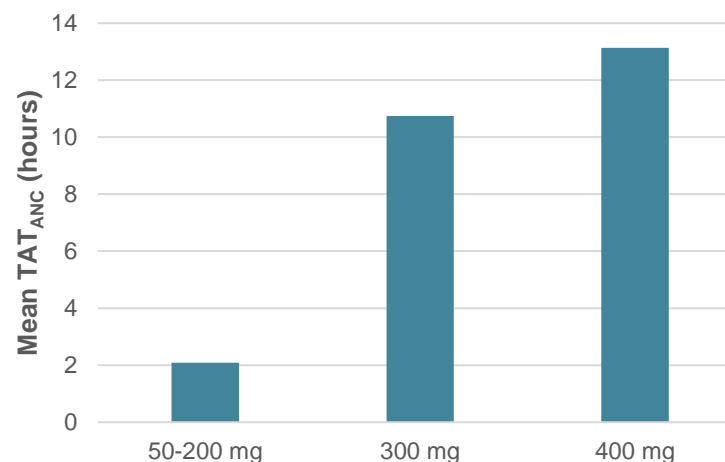
WHIM Phase 2 Trial Demonstrated Efficacy Using Endpoints in Ongoing Phase 3 Trial

6-Fold Increase in TAT_{ANC}

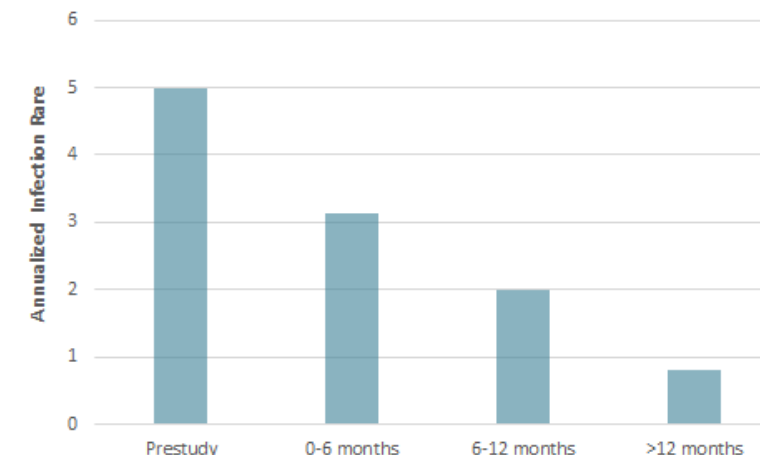
Phase 2 Study Design

- Intra-patient dose-escalation: safety, PK
- Endpoint: Clinically relevant blood measurement: **"Time above threshold for absolute neutrophil count" (TAT_{ANC})**
 - FDA & EMA agreement on use as P3 endpoint
 - Wart burden and infection rates also examined

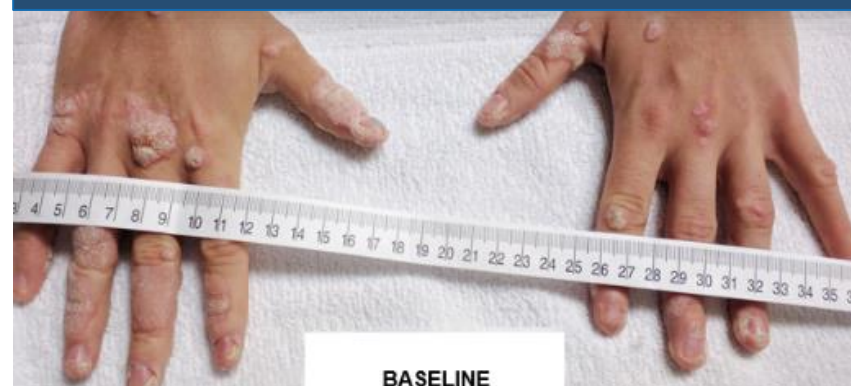
Major (>600%) Increase in Neutrophil Counts (TAT_{ANC})



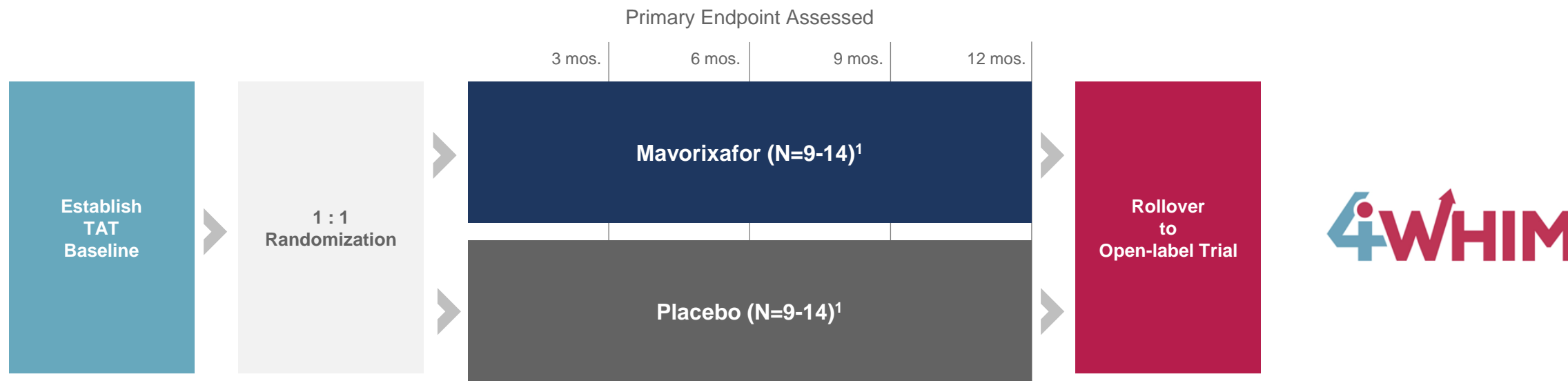
> 80% Reduction in Annualized Infection Rates



>75% reduction in the number of warts while on treatment



Mavorixafor Global Phase 3 Trial in WHIM - *Patients 12 years of age and older*



- **Primary Endpoint:** Biomarker of time above threshold for absolute neutrophil count (**TAT_{ANC}**); average of four assessment timepoints
- **Secondary Endpoints:** TAT_{ALC}, infections, wart burden, infection score composite, QoL assessment and others
- **Dosing:** 400mg QD in patients for subjects above 50 kg; 200 mg QD for those below 50 kg
- **Enrollment Complete:** Over-enrolled with 31 patients

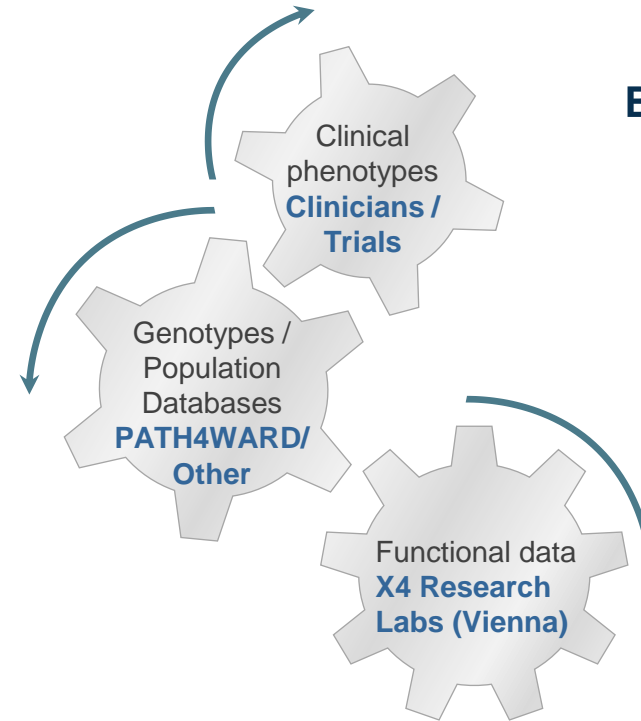
Phase 3 Top-line Data Expected in 4Q 2022

WHIM Prevalence: 1,000 – 3,700¹ in the U.S.

Market and AI Research Results: Estimated Prevalence



1. Company market research. Qessential market research, 2019 and IPM.ai artificial intelligence study, 2020.



Bench-to-Bedside Research

ASH 2021

**Newly identified D84H
CXCR4 mutation
suggests additional
~1,250-2,500 WHIM patients**

**4Q21
Initial
Data**

X4's success in increasing WHIM awareness and diagnosis

- Education and awareness: over-enrolled Phase 3 trial (3Q21)
- Free physician-initiated testing via PATH4WARD/Invitae partnership (update 4Q21)
- *New:* Free Patient-Initiated-Testing (PIT) with PATH4WARD
- *New:* Global harmonization patient registry - IPOPI
- *Ongoing:* Bench-to-bedside research leading to discovery of new mutations/patients
- *Ongoing:* Broadening genetic testing efforts with established neutropenia registries

Chronic Neutropenia Beyond WHIM

Mavorixafor antagonizes wild-type CXCR4; potential for broad treatment in neutropenias

What is Chronic Neutropenia (CN)?

Chronically immunocompromised patients due to sustained, low neutrophil counts (neutropenia)

The magnitude of neutropenia correlates with higher risk of severe infections and greater frequencies of infections

- Mild if ANC between 1,000 and 1,500/ μ L
- Moderate if ANC between 500 and 1,000/ μ L
- Severe if ANC <500/ μ L

Severe Infections and Certain Cancers Are Major Risks

- 9-fold risk of severe infections (defined as hospitalizations, needing IV antibiotics, and/or sepsis/death) in high-risk groups¹
- 10-30% life-time risk of developing AML²

Danish Study: Prevalence and Mortality Risk³

- 0.06% of population with neutropenia (<1,500 cells/microliter)
- All-cause mortality: 2.5-6.5X hazard ratio vs. non-neutropenic population

1. Bodey GP et al. Quantitative relationships between circulating leukocytes and infection in patients with acute leukemia. *Ann Intern Med*, 1966 2. Choi *Bone Mar Trans*. vol. 35. 2005. pp. 473-477. 3. Andersen et al, *J Intern Medicine*, 2016 Jun;279(6):566-75.

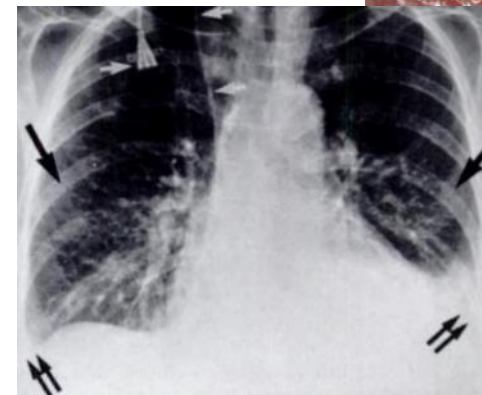
Skin Abscesses



Oral Ulcerations



Pneumonia

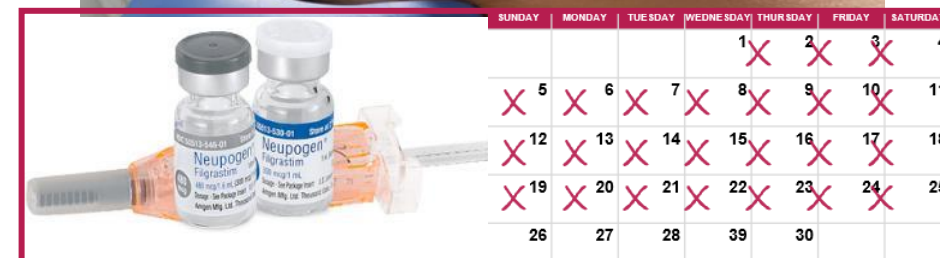


Chronic Neutropenia Management – High Unmet Needs

- **Injections of G-CSF: once or twice-daily, or three times per week** to maintain higher neutrophil levels (ANC) and reduce infections
 - Initial “dose titration” to aid with tolerability
 - Neutrophil target: ~1,000–2,000 cells/ μ L

Challenges for patients on G-CSF include

- 25% continue to experience severe bacterial infections while on chronic treatment¹
- Increasing risk of myelodysplastic syndromes (MDS) over years of treatment²
- ~70% have moderate or severe bone-pain impacting QoL³
- **No alternate therapies, except for bone marrow transplantation, are available**



Mavorixafor
Potential as an Oral,
Once-Daily Rx to
Reduce or Replace
G-CSF



U.S. G-CSF Market For Severe Chronic Neutropenia: Estimated \$120-460 Million

- **Injectable treatment** of growth factor
- Chronic treatment with G-CSF has been approved for certain types of **severe chronic neutropenia (ANC < 500 μ L)** and has been shown to reduces infections
- **>2,000 patients with chronic neutropenia estimated to be on regular G-CSF treatment in the US²**
- Annual cost of treatment at generic prices ranges **\$60,000 to \$230,000 per patient³**
 - Range depends on dose frequency and weight; some intermittent use

1. IQVIA Sales Audit – G-CSF

2. Based on research of U.S. claims data, EMR and SCNIR registry data; excludes all cancer/chemotherapy-related uses of G-CSF

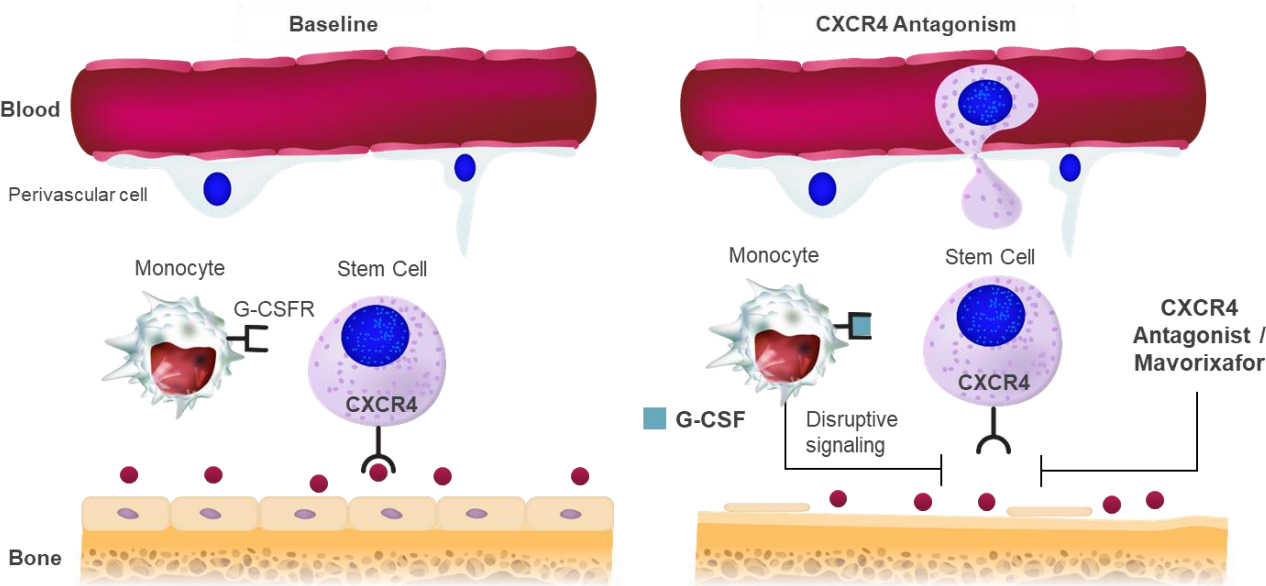
3. Based on labeled dosing for SCN and ASP for biosimilar products

Mavorixafor Has The Potential To Become Standard of Care in Neutropenias



Mavorixafor and G-CSF (Current Standard of Care) : Synergistic MOA via CXCR4 Signaling

- Both down-regulate CXCR4-signaling and cellular adhesion
- Both impact leukocyte maturation and trafficking
- Mavorixafor is oral vs. G-CSF injectable
- Mavorixafor has broader impact, potentially improving immunity beyond neutrophils



Mavorixafor vs G-CSF: New Standard of Care?

	Mavorixafor ¹ (Phase 2 - PoC)	G-CSF ² (Phase 3)
Patient Population	WHIM Syndrome	Severe Neutropenia: Congenital, Idiopathic, Cyclic
Chronic Increase in Neutrophils?	Yes	Yes
Infections	Reductions	Reductions
Safety	Well-tolerated	Thrombocytopenia, bone pain, & myelodysplastic syndrome risk
Dosage	Oral Fixed dose Once daily	Injection Dose-titration Up to twice daily

1. Dale et al. *Blood* (2020) 136 (26): 2994–3003.
2. Dale et al. *Blood* (1993) 81(10): 2496–2502.

Ongoing Phase 1b Trial: Assessing Mavorixafor in Patients with Chronic Neutropenia

Enrolling moderate and severe neutropenia patients

Phase 1b study: Proof of Concept in Broad CN Population

- Severe and moderate CN
- With or without genetic causes
- With or without G-CSF

Endpoints: Safety and tolerability, change in ANC (and other WBCs) vs. pre-treatment baseline

Goal: Achieve proof of concept to support FDA interactions regarding proposed registrational trial

*Studying mavorixafor
across broader chronic
neutropenia populations*

4Q21
Initial
Data

12 years or older; on or off G-CSF

Cohort A

- 14 days of treatment
- Congenital neutropenias
- Neutrophil count < 500 μ L

Cohort B

- 1 day of treatment
- Congenital, cyclic or idiopathic neutropenia
- Neutrophil count <1,000 μ L

Engaging with Primary Immune Deficiency Communities and Supporting Diagnosis

Sponsored Genetic Testing



MSL Deployment to engage concentrated, targeted physician population



Disease Education on WHIM and Waldenstrom's

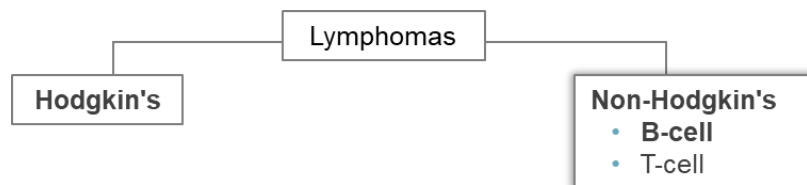
Ongoing Collaboration with key **Patient Advocacy Groups**



national neutropenia network

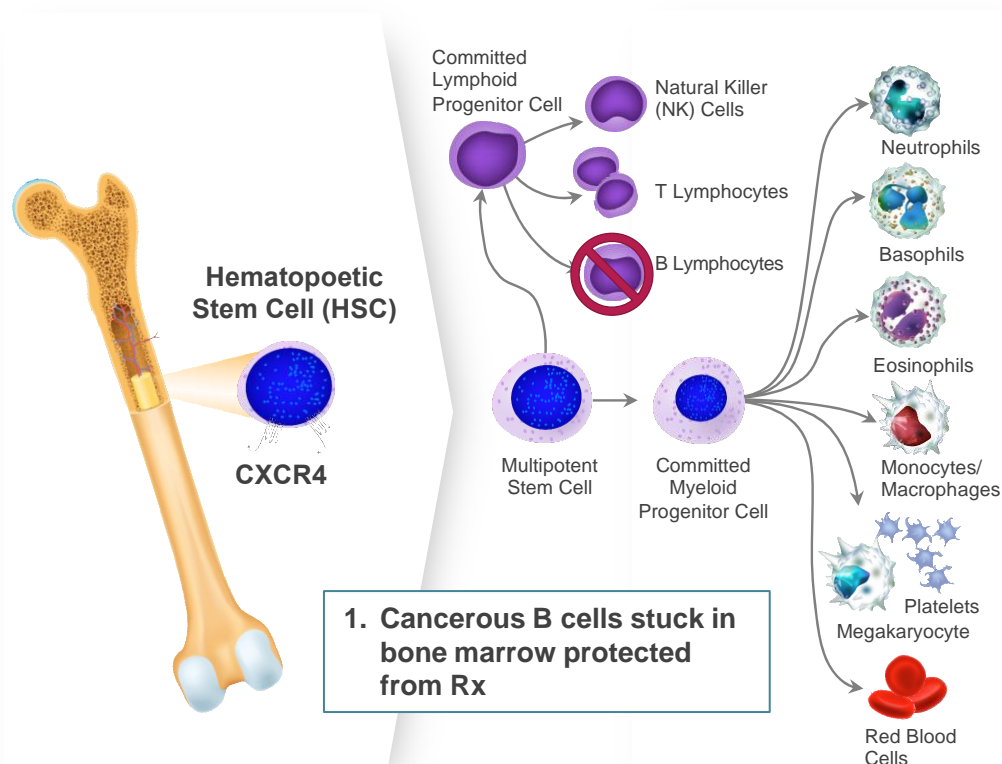


The Dysregulated Immune System: Lymphomas



Expansion

Maturation



1. Cancerous B cells stuck in bone marrow protected from Rx

2. Mavorixafor mobilizes cancer cells to increase susceptibility to Rx

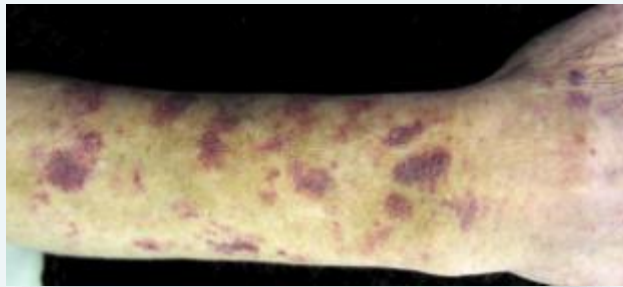
3. "Chemosensitization" by CXCR4 antagonist combination to improve efficacy in leukemias and lymphomas

Proof of Concept Established for CXCR4 Antagonism

B-Cell Lymphomas	U.S. Prevalence	CXCR4i Combinations in POC Studies
Diffuse Large B-Cell Lymphoma (DLBCL)	~20,000 ²	Plerixafor + Rituximab (Pre-Clinical)
Mantle Cell Lymphoma (MCL)	>4,000 ³	Plerixafor + Bortezomib (Pre-Clinical)
Waldenström's Macroglobulinemia (<i>non-CXCR4 mutation</i>)	~8,000-10,000 ⁴	Mav+SOC Pre-clinical in WT CXCR4
Waldenström's Macroglobulinemia (<i>CXCR4-mutation sub-population</i>)	~2,000-3,000 ⁴	Ulocuplumab + ibrutinib (Clinical) Mavorixafor + Ibrutinib (Clinical)

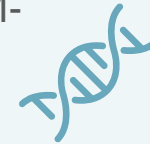
First PoC ongoing in CXCR4^{MUT} Waldenstrom's (patients with heaviest bone-marrow involvement)

A rare B-cell blood cancer of the bone marrow; most often (>90%) caused by mutations in the MYD88 gene, which is involved in innate immune response system



30-40%

Patients with identified WHIM-like CXCR4 mutations in addition to MYD88 mutation



~8-10

year survival rate post diagnosis^{1,2}

Signs and symptoms

- **Elevated IgM**
- Hyperviscosity syndrome
- Cryoglobulinemia – IgM clumping
- Pancytopenia, anemia
- Peripheral neuropathy
- Fever, night sweats, weight loss, fatigue



Limited Current Treatments

- Ibrutinib, zanubrutinib
- Chemo (bendamustine, R-CHOP)
- Rituximab
- Combinations and others

<50%

Mean progression-free survival (PFS) in CXCR4-mutation Waldenström's patients versus CXCR4-wild type³



Improvements in serum levels of IgM and hemoglobin

- *Reductions in IgM* changes correlated with Response definitions
- *Increases in hemoglobin* indicate reduction in cancer burden

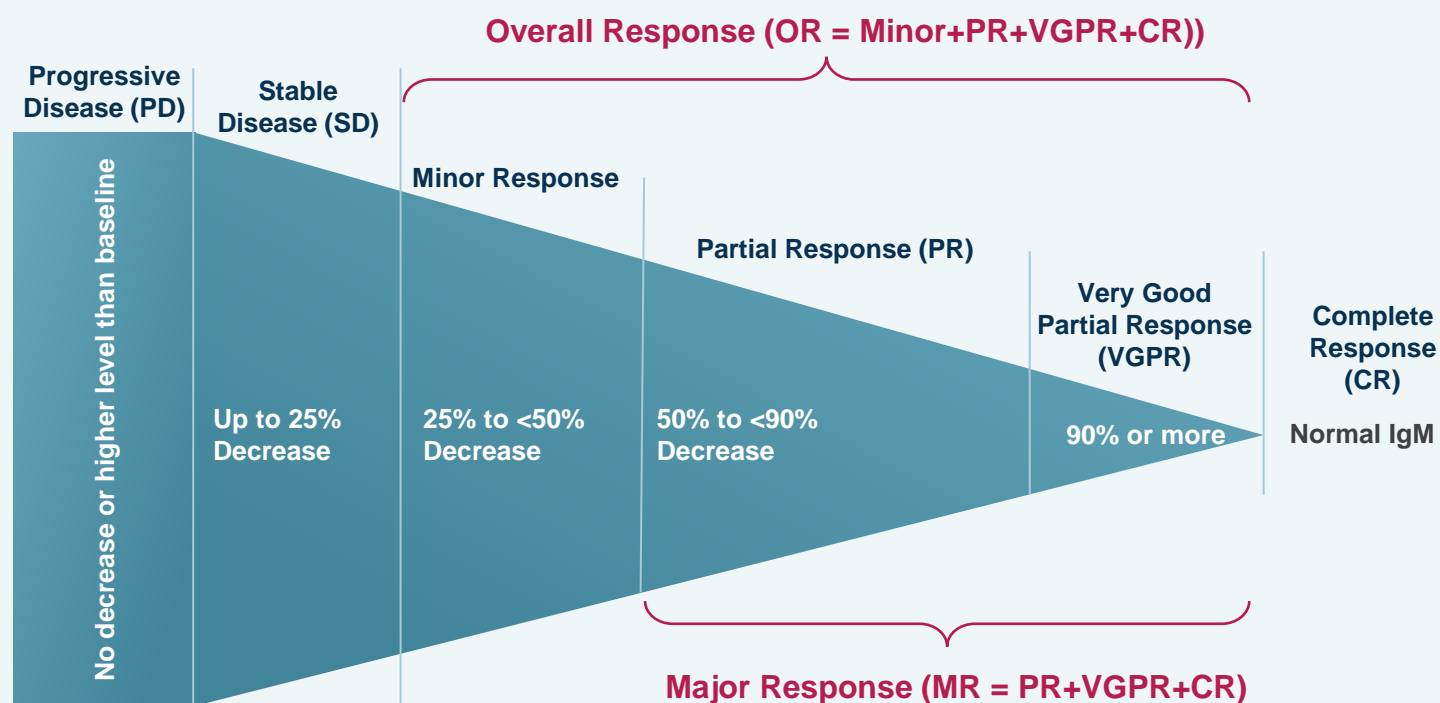
Improvements in Response Rates

- Responses correlate with progression free survival and overall survival

Treatment Response Criteria and Benchmarks in Waldenström's

Definition of treatment “response” In Waldenström’s: multiple components

① Decreasing serum IgM Levels (Normalized To Baseline)



② Stabilized Symptoms & Decrease in Extramedullary Disease (If Present)

Effect of Ibrutinib Monotherapy on IgM Levels in Double-Mutation Patients

	PREVIOUSLY TREATED ^{1,2}	FRONT LINE ³
Time to >50% Response (months)	4.7	7.3
Overall response at 6 months	85.7%	61.9%
Major Response at 6 months	38.1%	28.6%

1, 2. Treon, NEJM, 2015, Treon, JCO, 2020; 3. Treon, JCO, 2018.

Waldenström's Phase 1b Trial: Generating PoC in Patients with CXCR4^{MUT}

Inclusion: Patients with *MYD88* + *CXCR4* mutations who are naïve to ibrutinib

Design: Multi-national Phase 1b trial of mavorixafor in combination with ibrutinib (n=12 to 18)

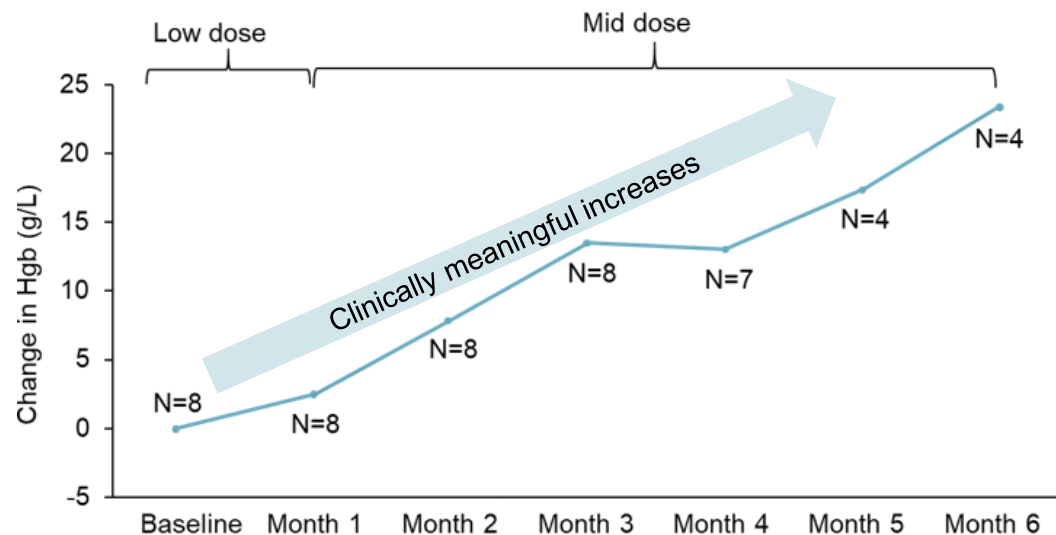
- **Inpatient dose-escalation:** cycles of 200 mg, 400 mg, and 600 mg QD
- **3 cohorts supporting dose selection of mavorixafor:**
 - Cohorts A & B: minimum of 6 patients enrolled in each
 - Cohort C (expansion): potential for additional 6 patients dosed up to 600 mg
- **Endpoints**
 - Safety, PK/PD
 - Assessments of serum IgM levels, hemoglobin, and clinical response



- Strategic collaboration with Leukemia & Lymphoma Society (LLS)
- Selected for LLS' Therapy Acceleration Program

EHA 2021: Mavorixafor + Ibrutinib Results in Clinically Meaningful Changes in IgM and Hemoglobin

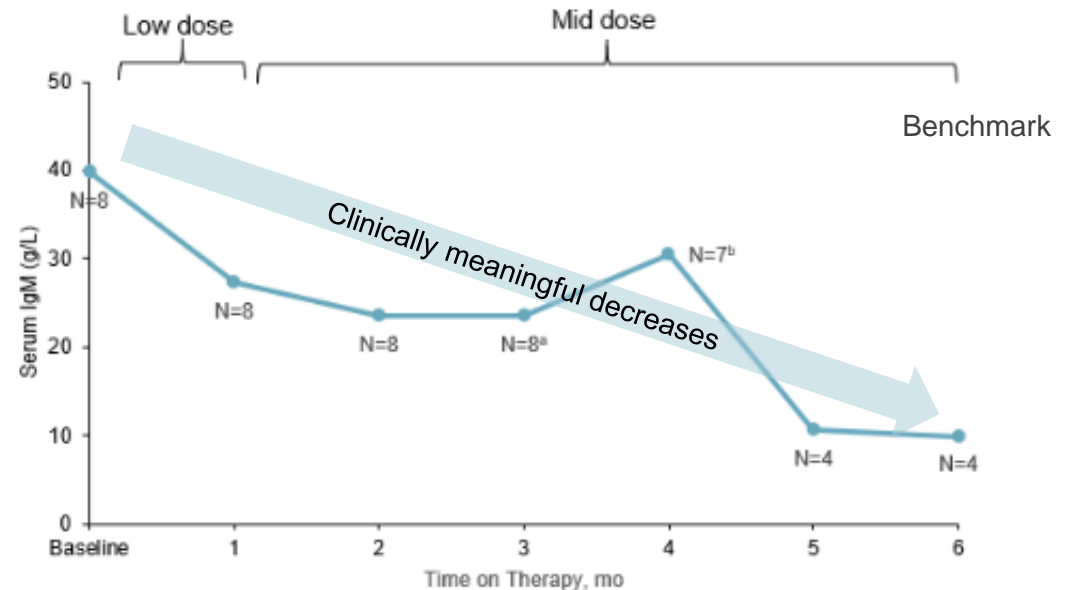
Median Change from Baseline Hemoglobin



At 6 months:

- Hemoglobin levels approached normal levels
- Key biomarker for resolution of anemia/fatigue and bone marrow health
- *Suggests reduction of cancer burden in the bone marrow*

Median Serum Levels IgM



At 6 months:

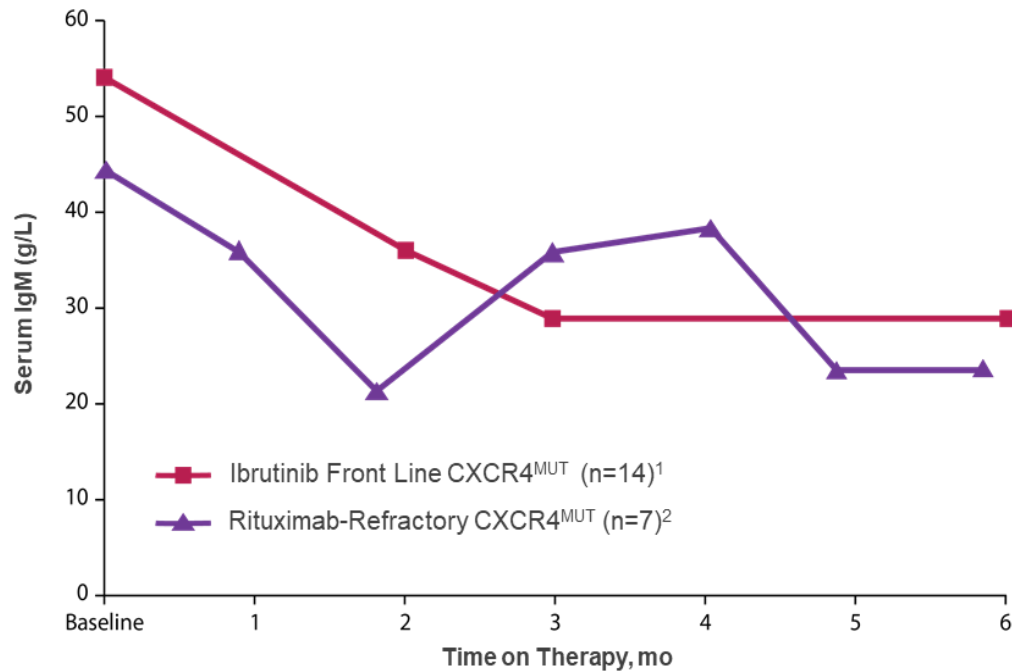
- 2 of 4 patients had >50% reduction from baseline
- 1 of 4 patients had absolute IgM levels within normal range
- *Early data compares favorably to historical data with ibrutinib alone*

^a IgM data of Patient 105-001 collected on May 10, 2021 were used to ensure 3 months' follow-up time.

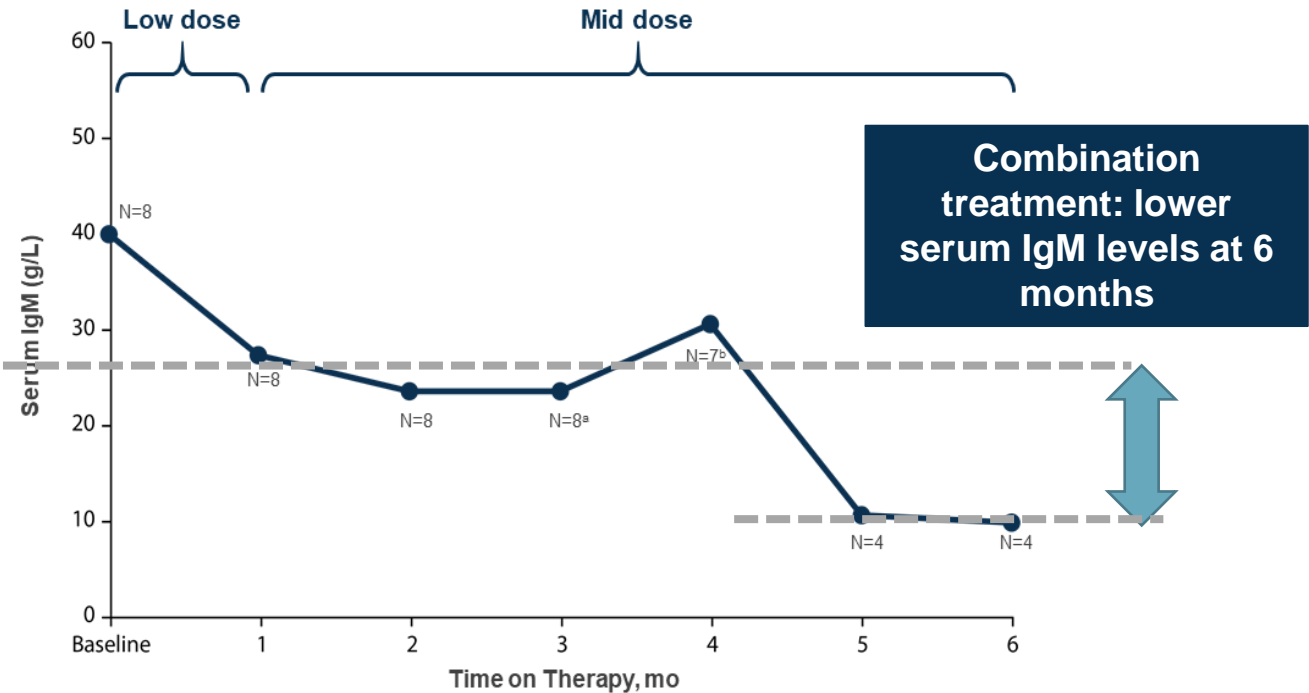
^b Participant 106-001 study treatment withheld due to an AE the week prior to month 4 IgM sample collection.

EHA 2021: Combo Data at Low and Mid-Doses Suggest More Deeper Reduction in IgM vs. Ibrutinib Monotherapy

Ibrutinib Monotherapy Median Serum IgM Levels



Mavorixafor + Ibrutinib Median Serum IgM Levels



1. Treon S., et. al *JCO* 2018 DOI: <https://doi.org/10.1200/JCO.2018.78.6426>
2. Dimopoulos et al, IWW9 Meeting, 2016, *Lancet Oncology*, 2017.

Waldenström's Phase 1b Data: Summary and Looking Ahead

- **Preliminary data suggest mavorixafor + ibrutinib brings clinically meaningful benefits in reduction in serum IgM and increases in hemoglobin**
- **At 6 months**, mavorixafor plus ibrutinib showed greater decreases in IgM in WM patients with CXCR4 mutations versus published ibrutinib monotherapy studies

	Ibrutinib Monotherapy	Mavorixafor + Ibrutinib
% IgM Drop from baseline	38-45%	60-75% (4-8 patient baseline)
≥50% Reduction	28-38%	50% (2 of 4 patients)

Targeting improvements in ibrutinib monotherapy: reductions in IgM and increased Overall Response (OR) and Major Response (MR) Rates in CXCR4^{MUT}

**4Q21
Additional
Data**

Anticipate additional data at all doses and initial response data (OR, MR)

Differentiated Next-Generation CXCR4 Antagonists Progressing into the Clinic

Supporting Corporate Growth

Candidate	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Expected Milestones
Mavorixafor	WHIM (Warts, Hypogammaglobulinemia, Infections and Myelokathexis) syndrome ¹	Phase 3				Top-line data 4Q 2022 NDA 1Q 2023
	Waldenström's Macroglobulinemia (WM)	Phase 1b				Additional data in 4Q 2021
	Chronic Neutropenia (SCN/CN) (Severe and Moderate)	Phase 1b				Initial data in 4Q 2021
X4P-002	Oncology indications	IND-enabling				IND in 2H 2022
X4P-003	Primary immuno-deficiencies (PID)	Advancing Dev Cand				

¹ Phase 2 open label extension (OLE) trial for WHIM ongoing

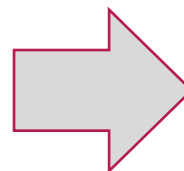
Catalyst-Rich Period Anticipated Next 12-18 months

- **3Q21 Milestones**

- ✓ WHIM Phase 3 trial enrollment complete with 31 patients
- ✓ ASH abstracts with data updates across all programs (100% acceptance)

- **4Q21 Expected Milestones**

- Response rates in Waldenstrom's Phase 1b
- Initial data in Chronic Neutropenia (CN) Phase 1b
- Long-term outcomes in ongoing WHIM Phase 2
- Bench to bedside research: WHIM prevalence/patient ID



- **Expected Milestones in 2022 and Beyond**

- Data & regulatory updates in CN and WM in 2H 2022
- Pipeline candidate IND filing in 2H 2022
- Pre-clinical PoC in lymphoma and neutropenia models in 2022
- Potential clinical study of mavorixafor in additional immunodeficiencies
- Continued reporting on expanding market opportunity in WHIM
- **WHIM Phase 3 top-line data in 4Q 2022**
- **Potential mavorixafor WHIM NDA filing in 1Q 2023**

Save the Date: December 16!

Investor/KOL Event to follow
the 2021 ASH Meeting



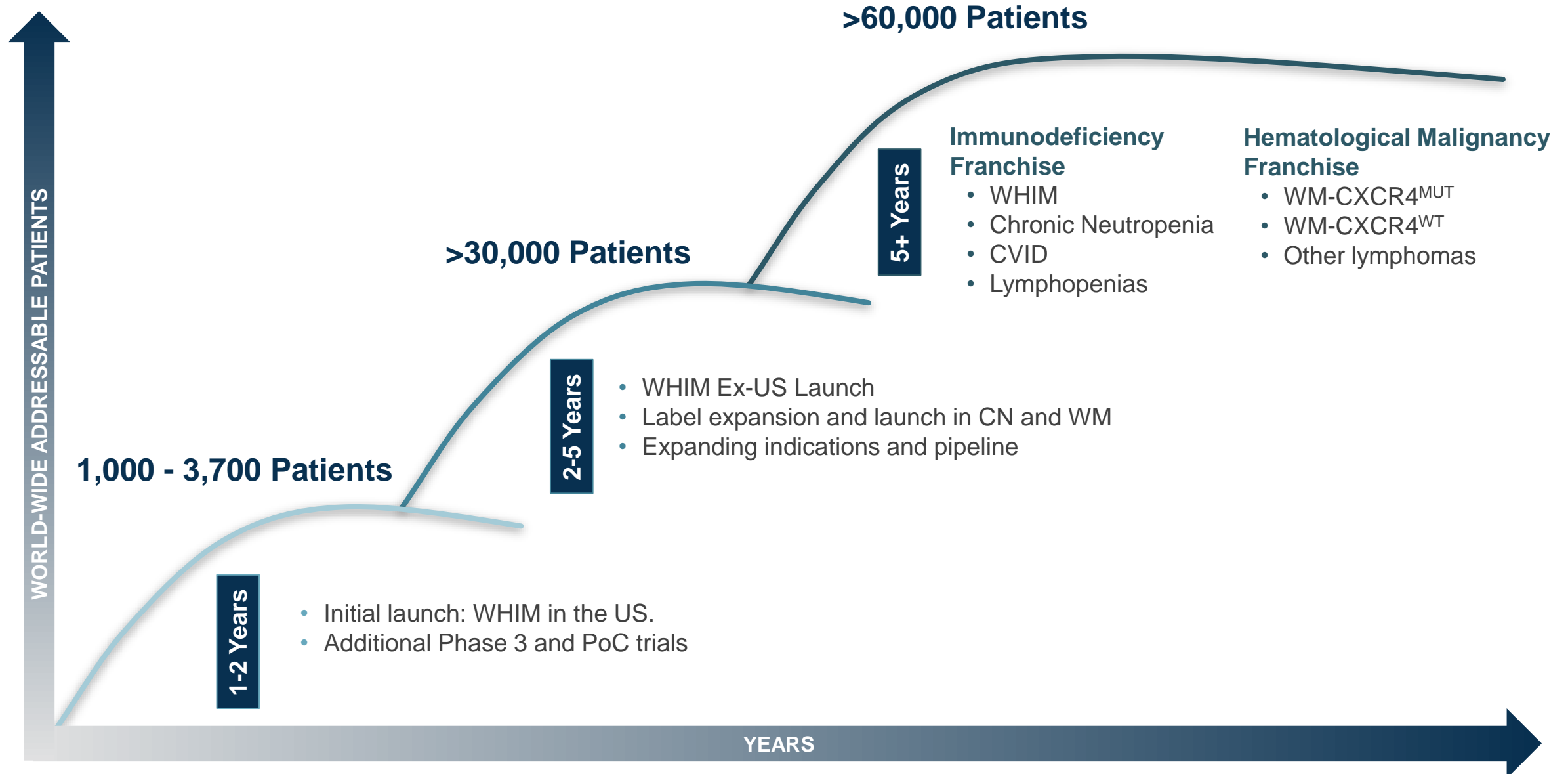
\$78M*

cash & equivalents**

*As of 9/30/2021; expected to fund operations into Q4 2022

**Excludes approximately \$19.5M in net proceeds raised from the sale of equity in November 2021

X4 Long Term Plan: Two Franchises Providing Rx for >60,000 Patients World-Wide





Thank you!



61 North Beacon Street
4th Floor
Boston, MA 02134

www.x4pharma.com

Selected Financial Highlights

\$78M¹

Cash Expected to Fund Operations into Q4 2022²

Share and Warrant Information:

**26.6M shares
outstanding**

(24.8M common shares
and 1.8M pre-funded
warrants)

**5.4M class B
warrants**

(expiry **30 days** post
WHIM P3 data)

**3.9M class A
warrants**

(2024 expiry)

Biotech-focused Institutional Shareholder Base

Analyst Coverage

cg/Canaccord
Genuity

COWEN

STIFEL

OPPENHEIMER

B | RILEY FBR

 **ROTH** Capital Partners

 **HCW**
H.C. WAINWRIGHT & CO.

BROOKLINE
CAPITAL MARKETS

¹ As of September 30, 2021, as reported in Company's form 10Q filed with the SEC on November 4, 2021; Excludes approximately \$19.5M in net proceeds raised from the sale of equity in November 2021

² As described in detail in our most recent Form 10-Q, our agreement with Hercules Capital, Inc. contains a minimum cash covenant that becomes effective on April 1, 2022. Based on our current financial projections, which do not include additional funding from third parties or potential amendments to the Hercules agreement, we expect that we would be in violation of this covenant in the second quarter of 2022, which could result in accelerated principal and interest payments due that could shorten our cash runway.

Time Above Threshold for Absolute Neutrophil Count (TAT_{ANC})

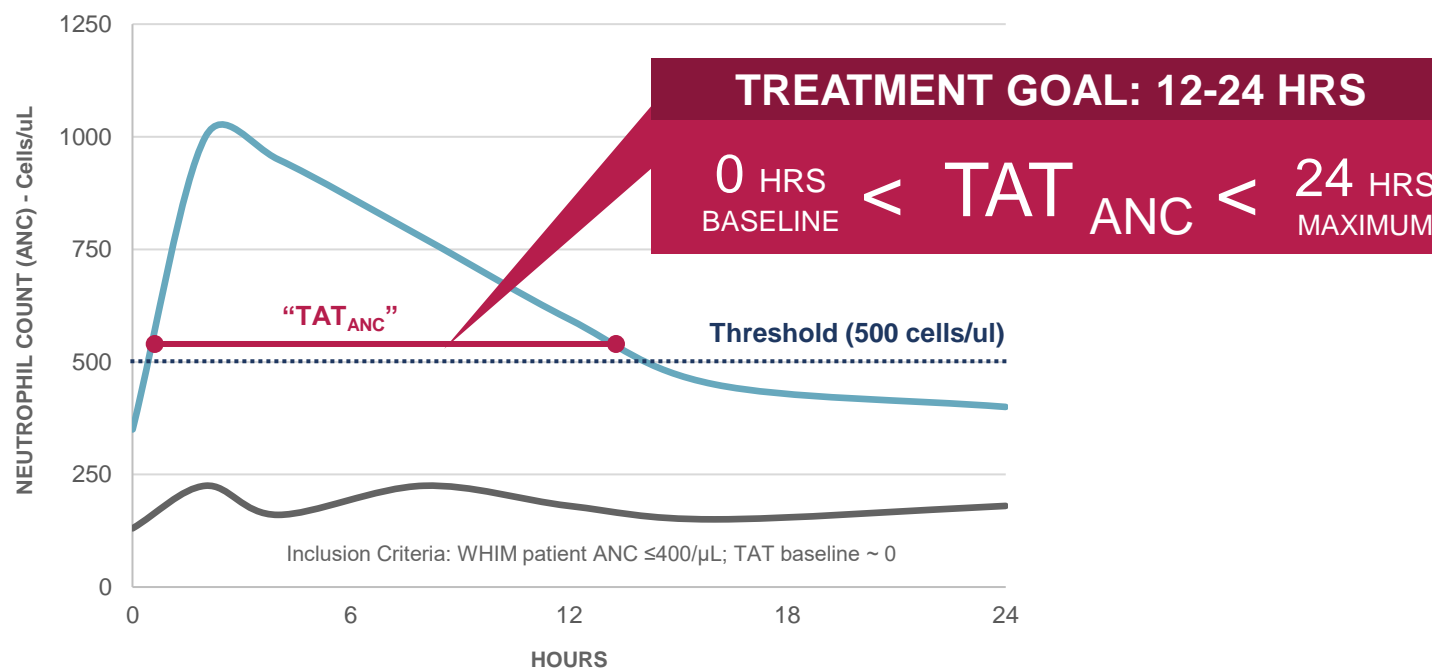
RATIONALE

SEVERE NEUTROPENIA IS WELL-ESTABLISHED TO CORRELATE WITH HIGH RISK OF SERIOUS/SEVERE INFECTIONS.

ISSUE: WHIM PATIENTS TYPICALLY HAVE SEVERE NEUTROPENIA ($ANC < 500$ CELLS/ μ L) ON A DAILY BASIS THROUGHOUT THEIR LIVES.

TREATMENT OBJECTIVE: INCREASE DAILY NEUTROPHIL COUNTS (ANC) ABOVE THRESHOLD ($ANC < 500$ CELLS/ μ L) AS MEASURED OVER 24 HOURS: TIME ABOVE THRESHOLD (TAT_{ANC})

ILLUSTRATIVE TRIAL ENDPOINT EXAMPLE



Phase 2 Study Results: At 400 mg QD, patients experience mean TAT_{ANC} of ~ 13 hours